

ABSTRACT**THERAPEUTIC COMPOSITIONS AND METHODS OF
TREATING GLYCOLIPID STORAGE RELATED DISORDERS**

5

- A method for treating a glycolipid storage-related disorder, comprising administering a therapeutically effective amount of an inhibitor of glycolipid synthesis in combination with an agent capable of increasing the rate of glycolipid degradation or in combination with bone marrow transplantation. Inhibitors of glycolipid synthesis include N-butyldeoxynojirimycin (NB-DNJ), N-butyldeoxygalactonojirimycin (NB-DGJ) or N-nonyldeoxynojirimycin (NN-DNJ). Glycolipid storage-related disorders include Gaucher disease, Sandhoff's disease, Fabry's disease, Tay-Sach's disease, Niemann-Pick C storage disease, GM1 gangliosidosis, genetic disorders in which neuronal glycolipid accumulation contributes to disease pathology.
- 10
- 15

PCT/US2003/030325